Personalised Medicine

ESF Workshop on Health Research Strategic Needs in Europe
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1. “Can Europe lead the global way?”

2. Challenges, bottlenecks, gaps and needs?
1. “Can Europe lead the global way?”

European activities and milestones?
a. European Science Foundation (ESF)
ESF Forward Look

Personalised Medicine for the European Citizen
Towards more precise medicine for the diagnosis, treatment and prevention of disease (pM)

ESF Position Paper

May 2011

Technology

ESF Position Paper

19-20 Sept 2011
London, UK

Disease Summit

18-20 Oct 2011
The Hague, NL

1) CV & metabolic diseases
2) Oncology
3) Rare Diseases

“Big picture” Summit on clustered issues

13-14 Feb 2012
Dubrovnik, HR

Identify grand challenges and recommendation

In-depth expert interviews

Stakeholder conference

18 April 2012
Rome, IT

Consensus discussion on Grand Challenges and overall recommendations
b. CSA-PerMed
FP7 CSA - PerMed
Personalized Medicine 2020 and Beyond – Preparing Europe for Leading the Global Way

Consortium: Research and Health Ministries (funding bodies)
Connected to other key European initiatives in Personalized Medicine (e.g. ESF, PHGEN, EHFG, EAPM, EPEMED, HOPE)
Aim: Strategic Research and Innovation Agenda (SRIA) for Europe

1st Workshop of stakeholders March 27/28th 2014 in Berlin
Parallel Forum at the European Health Forum Gastein (EHFG), October 1-3, 2014
Discussion SRIA & PerMed Roundtable, Barcelona, March 25-26, 2015

Webpage: http://www.permed2020.eu
c. European Health Forum Gastein (EHFG)

d. European Alliance for Personalised Medicine (EAPM)
Specialised Treatment for Europe’s Patients – „STEPS“

- STEP 1: Ensuring a regulatory environment which allows early patient access to novel and efficacious personalised medicine (PM)

- STEP 2: Increasing R&D for PM, while also recognising its value

- STEP 3: Improving the education and training of healthcare professionals

- STEP 4: Supporting new approaches to reimbursement and HTA, required for patient access to PM

- STEP 5: Increasing awareness and understanding of PM

EAPM believes that achieving these goals will improve the quality of life for patients in every country in Europe.

An introduction to EAPM, its Policy Taskforces and STEPS campaign
2. Challenges, bottlenecks, gaps and needs?
(Public) Health moves towards Personalised Medicine...

Public Health Genomics (PHG) – Personal Health and Care
The BIG4HEALTH 😊
- four key research policy areas for Europe ...

1. decision-supporting tools

2. “big data”

3. ownership

4. health systems
1. decision-supporting tools

- HTA 3.0 (assumption non-linearity and “personal evidence”)

- systematic early dialogue/PPP (e.g. LAL model, MAPPs), best practice of PPP = IMI

- “just in time” interventions (JITs)

- orphan drug model & pilots (e.g. Germany: “Heilversuch” with N=25) / RoI

- drug/theranostics/CDx/IVD versus Medical Device ... (use of) health information (HI)

- “virtual twin”: in silico ”try and error“ (simulations, artificial learning)
2. “big data”

• **N=1 trials:** “I am my own reference point”

• **N=all trials:** mission impossible (“big data” will always be incomplete)

• **unstructured** (and structured) data for unknown future purposes (more than just data linkage or open access)

• **validation, standardization:** mission impossible (always a “momentum”)

• “**incidental findings**”/noise: all findings are important, we just cannot interpret them (yet): “junk versus garbage”

• **health information will always be “messy”/**chaotic: what (not why) is good enough in most cases! **Correlation** versus causality ...
3. Ownership

• “I am the owner of my data”: personal ownership (property based, excluding right, paternalistic) vs. citizen ownership/control (broader, social right, shared right, democratic)

• from informed consent (blanket or broad) & privacy issues to data-users accountability: “trust & trusts”! (... to guarantee data security is dishonest!)

• “big data” meet governance of information via algorithm providers (QM): rules of impartiality, confidentiality, competence (interpretation of data) and professionalism

• Health Data Cooperatives (balance between public good – personal benefit, no monetary incentives for individuals!)
4. health systems

• “good governance” – “good” implementation of “good” health policies (e.g. in Europe cross-border directive, “bottom-up” policies)
• WHO-EU Regional office (Tallinn, 2008): six system building blocks
The next step???

ERA-net on Personalised Medicine!
Thank you! ¡Gracias! Arigato! Shukuria! Viya! Bolzín! Merci!